

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
12 February 2004 (12.02.2004)

PCT

(10) International Publication Number
WO 2004/013310 A2

(51) International Patent Classification⁷: C12N

(74) Agents: BOOTH, PAUL M et al.; HELLER EHRMAN
WHITE & MCAULIFFE LLP, 1666 K Street, N.W., Suite
300, Washington, DC 20006-1228 (US).

(21) International Application Number:
PCT/US2003/024587

(22) International Filing Date: 6 August 2003 (06.08.2003)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/401,029 6 August 2002 (06.08.2002) US

(71) Applicant (for all designated States except US): IN-
TRADIGM CORPORATION [US/US]; 12115 Parklawn
Drive, Suite K, Rockville, MD 20852 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): SCARIA, Puthup-
parampil, V. [US/US]; 9602 Swallow Point Way, Mont-
gomery Village, MD 20886 (US). WOODLE, Martin, C.
[US/US]; 8205 Beech Tree Road, Bethesda, MD 20817
(US). LU, Patrick, Y. [US/US]; 17093 Briardale Road,
Rockville, MD 20855 (US). TANG, Qingquan [US/US];
31 Longmeadow Drive, Gaithersburg, MD 20878 (US).
XU, Jun [CN/US]; 18120 Coachmans Road, Germantown,
MD 20874 (US). XIE, Frank, Y. [CN/US]; 13921 Rock-
ingham Road, Germantown, MD 20874 (US).

(81) Designated States (national): AE, AG, AL, AM, AT, AU,
AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU,
CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH,
GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC,
LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW,
MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC,
SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA,
UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

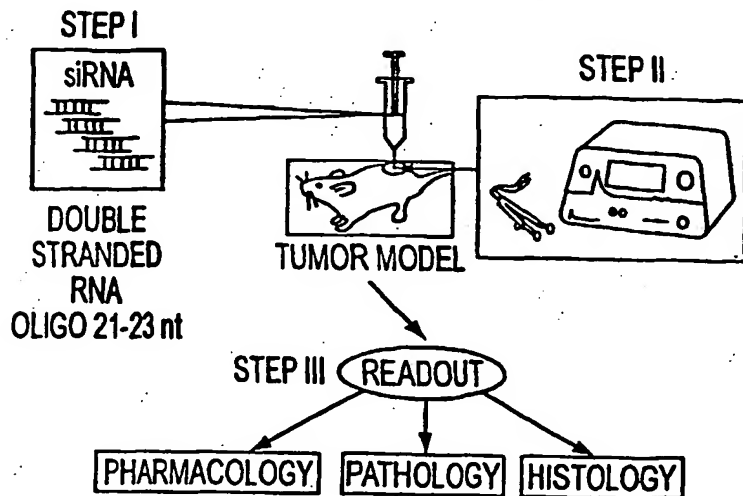
(84) Designated States (regional): ARIPO patent (GH, GM,
KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW),
Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),
European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE,
ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO,
SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM,
GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— without international search report and to be republished
upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.

(54) Title: METHODS OF DOWN REGULATING TARGET GENE EXPRESSION IN VIVO BY INTRODUCTION OF INTER-
FERING RNA



(57) Abstract: Methods and compositions are provided for down regulation of target gene expression in vivo by RNA interference. The methods are useful for target discovery and validation of gene-based drug development, and for treatment of human diseases.

WO 2004/013310 A2